

PODIUM SESSION III:
CANCER OUTCOMES RESEARCH

CA1

DISCORDANT DIAGNOSES IN SARCOMA, GIST AND DESMOID TUMOR IN FRANCE: RESULTS FROM THE NETWORK RRePS

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OBJECTIVES: Major discordant diagnoses may have strong impact on therapeutic management. So, identification of major discordant diagnoses and predictive factors were conducted in sarcoma patients. **METHODS:** A multicenter analysis was performed retrospectively from the prospective cohort of sarcoma patients. Inclusion criteria were patients with a diagnosis of sarcoma in 2010 and with a second opinion performed within the network RRePS (Réseau de Référence en Pathologie des Sarcomes supported by the French NCI). Major discordant diagnoses were defined as: sarcoma vs benign lesion, sarcoma vs malignant non sarcoma tumor, gastrointestinal stromal tumors (GIST) vs non GIST, and desmoid tumor vs non desmoid tumor. Patient and disease characteristics were described. Logistic regressions were used in order to define predictive factors of major discordance. **RESULTS:** 3621 patients were included in the study. 438 patients (12%) had a major discordant diagnoses: sarcoma versus benign lesion (or conversely) in 155 patients (58%); sarcoma instead of malignant non sarcoma tumor (or conversely) in 103 patients (24%); gastrointestinal stromal tumors (GIST) instead of non GIST in 48 patients (11%); desmoid tumor instead of non desmoid tumor in 28 patients (6%) and other (4%). Major diagnostic discordances risks were higher (i) for malignant non sarcoma tumors compared to GIST, liposarcoma, and other sarcoma histological subtypes ($p=0.004$); (ii) for patients who had a previous cancer ($p=0.03$); (iii) for limb localization compared to trunk ($p=0.004$); (iv) when the second opinion was requested by the initial pathologist ($p<0.01$). **CONCLUSIONS:** This study reported that sarcoma instead of benign lesion (or conversely) is the major discordant diagnosis in sarcoma patients implying that: (i) patients who should not be treated received anticancer therapy; (ii) treatments are potentially delayed for patients who should be rapidly treated. Economics evaluations are in progress in order to advise health care administrators regarding systematic second reviews in the management of sarcoma.

CA2

APPLYING A VALUE-BASED PRICE ACROSS DIFFERENT DISEASE AREAS

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OBJECTIVES: Value-based pricing is currently a topic of much interest, and there is wide-spread speculation as to how it will be implemented. The aim of this research was to address the question as to how reimbursement bodies will consider value-based pricing with regard to pharmaceuticals that can be used in multiple indications. Will there be separate prices, or an average price weighted by the population size of each different population? If so, then would the price have to be re-assessed each time the drug is approved for a new indication? **METHODS:** The National Institute for Health and Clinical Excellence (NICE) in the UK was used as an example to identify those therapies that have been appraised for multiple indications. All NICE technology assessments for cancer treatments published since January 2005 were reviewed. Therapies used in different indications were identified and ICERs from these appraisals were extracted. **RESULTS:** In total, 12 different treatments were identified, spanning sixteen different indications within cancer. Of these, five (cetuximab, docetaxel, imatinib, pemetrexed and trastuzumab) had ICERs that were either side of the £30,000 per QALY threshold across different disease areas. For example, the ICERs associated with cetuximab were found to vary from £6,400 (squamous cell cancer of the head and neck) to £90,000 (colorectal cancer) per QALY. These data suggest that there may be a large discrepancy when considering value-based pricing across different cancer populations. **CONCLUSIONS:** Across indications, the ICERs for a single therapy were found to vary dramatically. Considering that almost half of therapies identified had ICERs either side of the £30,000 per QALY threshold, these results highlight the potential problem associated with labelling a pharmacologic treatment with a single value-based price. Therefore, a single price may not be appropriate, and alternative methods should be considered by reimbursement bodies.

CA3

USE OF HEALTH CARE ADMINISTRATIVE DATABASES TO ESTIMATE THE BURDEN OF BREAST CANCER

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OBJECTIVES: To assess the epidemiologic and economic burden of breast cancer (BC) at any stage from a large population-based study. **METHODS:** Lombardy Region includes 9.9 million individuals. Eligible patients were identified through a data warehouse (DENALI), which matches with a probabilistic linkage demographic, clinical and economic data of different Healthcare Administrative databases. All female individuals who had a first hospital discharge with a IDC-9 CM code 174.XX from 1st January 2004 were selected and followed up to 5 years. These subjects were considered incident patients since they had no cause-specific hospitalizations during 2000-2003 period. We calculated yearly incidence, mortality and health care costs (hospitalizations, drugs and outpatient examinations/visits)

from the National Health Service's perspective (NHS). **RESULTS:** A total of 50,868 eligible subjects (mean age \pm SD equals to 62.5 \pm 14.2) were identified. Incidence patients were homogeneously distributed during the observational period: 20.5% in 2004, 20.1% in 2005, 19.7% in 2006, and 19.4% and 20.2 in 2007 and 2008. During the 2005-2008 period, the mean cost/patient-year for incident and prevalent cases were: 12,973€ versus 4,428€ in 2005, 13,847€ versus 4,237€ in 2006, 14,742€ versus 4,400€ in 2007, 15,671€ versus 4,336€ in 2008. Of the total cost of incident patients, hospitalizations were the driver (70%), with drugs and outpatient claims contributing to 16.3% and 16.2%, respectively. The driver of total costs in prevalent patients was drugs (41.1%), followed by hospitalizations and outpatients claims, contributing to 37.1% and 21.8%, respectively. Overall 46.3 deaths/1,000 patients-year were estimated with a probability of survival equals to 80% after 5 years from the index date. **CONCLUSIONS:** The high epidemiological and economic burden of BC, indicates the primary importance in monitoring the developing of the disease from the NHS's perspective. DENALI shows to be an efficient instrument combining administrative databases to accurately estimate the burden of BC.

CA4

A COMPARISON OF PATIENT AND GENERAL-POPULATION UTILITY VALUES FOR ADVANCED MELANOMA IN HEALTH ECONOMIC MODELLING

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OBJECTIVES: Health-related quality of life (HRQL) is an essential part of health technology assessment; without this Quality Adjusted Life Years cannot be calculated. The objective of this study was to compare utilities calculated for patients with advanced melanoma in the Phase III clinical trial for ipilimumab (MDX010-20) with utilities produced by vignettes for advanced melanoma valued by the general population. A comparison was also made between standard 'progression-based' utilities and those based on the time elapsed between the utility measurement and the patient's death. **METHODS:** Utilities from the trial were generated using the EORTC-8D and SF-6D preference-based measures. Analyses by progression status and time to death were conducted on patient-level data, and the prognostic value of the methods was assessed. Patient-level results were then compared with the utilities derived for progressive and non-progressive disease in a separate vignette study. **RESULTS:** SF-6D and EORTC-8D showed a substantial decrease in utility in the 180 days before the patient's death (from 0.83 to 0.63 and from 0.66 to 0.51, respectively), which is not consistent with the use of standard Markov progression-based, health-state modelling. Time to death showed a lower Root Mean Squared Error and higher R² when used to predict patient utility, demonstrating that they produce a more accurate assessment of HRQL. Utilities taken from vignettes showed a larger decrease on disease progression (from 0.77 to 0.59) than either the generic SF-6D (from 0.64 to 0.62) or condition-specific EORTC-8D (from 0.80 to 0.76). **CONCLUSIONS:** Although most oncology modelling is based around disease progression, this may not always be appropriate because the time to a patient's death appears to be a more accurate predictor of HRQL. This has implications for the analysis of utility information in future cost-effectiveness studies as well as the modelling methods used for oncology treatments and health technology assessments.

PODIUM SESSION III:

CLINICAL OUTCOMES STUDY METHODOLOGICAL CHALLENGES

CL1

A MULTICRITERIA APPROACH FOR EVALUATING HEALTH-RELATED QUALITY-OF-LIFE

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OBJECTIVES: The main aim of this paper is to develop a new approach for analyzing EQ-5D data based on the principles of multicriteria analysis. A data set of 73 patients after lumbar discectomy has been used in order to illustrate the applicability of the model. **METHODS:** The proposed approach is an ordinal regression model for measuring and analyzing EQ-5D data. The main objective of the method is the aggregation of individual judgments into a collective value function. The proposed approach provides a series of normalized average indices for each one of the dimensions of the EQ-5D instrument, as well as a set of perceptual maps. These results include: average satisfaction, demanding, and improvement indices, as well as action and improvement diagrams. **RESULTS:** The analysis of the sample revealed a relatively high satisfaction level (84.65%) for the general health status (VAS). Furthermore, the analysis of the EQ-5D questions shows that patients are very satisfied regarding the dimensions of self-care (95.67%) and pain/discomfort (95.63%). On the other hand, the lowest health status score refers to the dimension of mobility (78.24%). The weights of these five dimensions are additional results of the proposed method. Based on this particular sample, the results show that the most important factor is the anxiety/depression (32.11%), following by the dimensions of mobility (21.23%) and usual activities (19.16%). Combining these results, the action diagram reveals a gap regarding the perceptions of these particular patients and proposes mobility and anxiety/depression as critical health status dimensions. **CONCLUSIONS:** The main advantage of the method is the ability to consider the qualitative (ordinal) nature of the input information. In addition, the provided results are rich enough to give a clearer view about the patient's health status.

CL2

STATISTICAL CONSIDERATIONS IN ESTIMATING SURVIVAL FOR ECONOMIC EVALUATIONS IN ONCOLOGY

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